



4164-01-P

DEPARTMENT OF HEALTH AND HUMAN SERVICES

Food and Drug Administration

[Docket No. FDA-2018-N-0001]

Utilizing Innovative Statistical Methods and Trial Designs in Rare Disease Settings; Public Workshop

AGENCY: Food and Drug Administration, HHS.

ACTION: Notice of public workshop.

SUMMARY: The Food and Drug Administration (FDA) is announcing the following 1-day public workshop entitled "Utilizing Innovative Statistical Methods and Trial Designs in Rare Disease Settings." This workshop is convened by the Duke-Robert J. Margolis, MD, Center for Health Policy at Duke University and supported by a cooperative agreement with FDA. The purpose of the public workshop is to bring rare disease stakeholders together to discuss the challenges associated with the development and regulatory decision-making for rare disease treatments and to also discuss promising study designs and analytical methods that can help overcome these challenges.

DATES: The public workshop will be held on March 19, 2018, from 9 a.m. to 5 p.m. Eastern Daylight Time (EDT). See the SUPPLEMENTARY INFORMATION section for registration date and information.

ADDRESSES: The public workshop will be held at the DoubleTree by Hilton Hotel Washington DC-Silver Spring, 8727 Colesville Rd, Silver Spring, MD 20910. For additional travel and hotel information, please refer to the Duke Margolis Center for Health Policy website

at: <https://healthpolicy.duke.edu/events/innovative-tools-and-statistical-methods-treatment-development-rare-disease-settings>.

FOR FURTHER INFORMATION CONTACT: Robyn Bent, Center for Drug Evaluation and Research, Food and Drug Administration, 10903 New Hampshire Ave., Silver Spring, MD 20993, 240-402-2572, Robyn.Bent@fda.hhs.gov.

SUPPLEMENTARY INFORMATION:

I. Background

Rare disease settings pose several significant challenges for clinical research, drug development, and regulatory review. Small population sizes, possible limited scientific understanding of the disease of interest, and a lack of market incentives often preclude more traditional clinical trial or analytical approaches from being pursued. To help collaboratively address these barriers, FDA is working with stakeholders to solicit feedback on promising designs and methodologies for use in the development of rare disease treatments that can form the basis of formal guidance documents.

II. Topics for Discussion at the Public Workshop

During the public workshop, speakers and participants will discuss a range of tools and methods that can be used in the development of treatments for rare diseases and small patient populations. The meeting will include both presentations by panelists and dedicated time for questions and comments from attendees. Topics will include: master protocols, use of external controls in single-arm trials, analytical tools for trials with multiple or novel endpoints, and best practices for leveraging Bayesian statistics and adaptive study designs.

III. Participating in the Public Workshop

Registration: To register for the public workshop, visit the following website: <https://healthpolicy.duke.edu/events/innovative-tools-and-statistical-methods-treatment-development-rare-disease-settings>. If you are unable to attend the meeting in person, you can register to view a live webcast of the meeting. There will be no onsite registration. Please provide complete contact information for each attendee, including name, title, affiliation, address, email, and telephone.

Registration is free and based on space availability, with priority given to early registrants. Persons interested in attending this public workshop must register by 5 p.m. EDT on Thursday, March 15, 2018. Early registration is recommended because seating is limited; therefore, FDA may limit the number of participants from each organization.

If you need special accommodations due to a disability, please contact Sarah Supsiri at the Duke-Margolis Center for Health Policy (phone: 202-791-9561, email: sarah.supsiri@duke.edu) no later than March 12, 2018.

Streaming webcast of the public workshop: This public workshop will also be webcast. Archived video footage will also be available at the Duke-Margolis website following the workshop (<https://healthpolicy.duke.edu/events/innovative-tools-and-statistical-methods-treatment-development-rare-disease-settings>). Persons interested in viewing the live webcast must register online before 5 p.m. EDT on March 18, 2018 (see *Registration*). Early registration is recommended because webcast connections are limited. Organizations are requested to register all participants, but to view using one connection per location whenever possible. Webcast participants will be sent technical system requirements in advance of the event. Prior to

joining the streaming webcast of the public workshop, it is recommended that you review these technical system requirements.

Transcripts: Please be advised that transcripts will not be available.

Other Issues for Consideration: A 1-hour lunch break is scheduled, but food will not be provided. There are multiple restaurants within walking distance of the DoubleTree by Hilton Hotel, 8727 Colesville Rd, Silver Spring, MD 20910.

All event materials will be provided to registered attendees via email prior to the workshop and will be publicly available at the Duke-Margolis Center for Health Policy website (<https://healthpolicy.duke.edu/events/innovative-tools-and-statistical-methods-treatment-development-rare-disease-settings>).

Dated: February 7, 2018.

Leslie Kux,

Associate Commissioner for Policy.

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